

117TH CONGRESS
1ST SESSION

H. R. 5416

To amend the Federal Food, Drug, and Cosmetic Act to establish additional authorities of the Food and Drug Administration regarding the conduct of pediatric investigations of molecularly targeted drugs to treat cancer, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

SEPTEMBER 29, 2021

Mr. BUTTERFIELD (for himself, Mr. McCaul, and Ms. Wild) introduced the following bill; which was referred to the Committee on Energy and Commerce

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to establish additional authorities of the Food and Drug Administration regarding the conduct of pediatric investigations of molecularly targeted drugs to treat cancer, and for other purposes.

1 *Be it enacted by the Senate and House of Representa-
2 tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the “Give Kids a Chance
5 Act”.

1 SEC. 2. RESEARCH INTO PEDIATRIC USES OF DRUGS; ADDI-

2 TIONAL AUTHORITIES OF FOOD AND DRUG

3 ADMINISTRATION REGARDING MOLECU-

4 LARLY TARGETED CANCER DRUGS.

5 (a) IN GENERAL.—

10 (A) by redesignating subparagraphs (B)
11 and (C) as subparagraphs (C) and (D), respec-
12 tively; and

15 “(A) IN GENERAL.—For purposes of para-
16 graph (1)(B), the investigation described in this
17 paragraph is (as determined by the Secretary)
18 a molecularly targeted pediatric cancer inves-
19 tigation of—

“(i) the drug or biological product for which the application referred to in such paragraph is submitted; or

23 “(ii) the active ingredient or ingredients
24 of such drug or biological product in
25 combination with—

1 “(I) an active ingredient of a
2 drug or biological product for which
3 there is in effect an exemption for in-
4 vestigational use under section 505(i)
5 that was submitted by the person sub-
6 mitting the application referred to in
7 paragraph (1)(B); or

8 “(II) an active ingredient of a
9 drug for which an approval under sec-
10 tion 505 is in effect, or an active in-
11 gredient of a biological product for
12 which an approval under section 351
13 of the Public Health Service Act is in
14 effect.

15 “(B) ADDITIONAL REQUIREMENTS.—

16 “(i) DESIGN OF INVESTIGATION.—A
17 molecularly targeted pediatric cancer inves-
18 tigation referred to in subparagraph (A)
19 shall be designed to yield clinically mean-
20 ingful pediatric study data, gathered using
21 appropriate formulations for each age
22 group for which the study is required, re-
23 garding dosing, safety, and preliminary ef-
24 ficiency.

1 “(ii) PURPOSE OF INVESTIGATION.—

2 The purpose of a molecularly targeted pe-
3 diatric cancer investigation referred to in
4 subparagraph (A) shall be—

5 “(I) in the case of such an inves-
6 tigation conducted with respect to a
7 drug or biological product referred to
8 in clause (i) of such subparagraph, to
9 inform potential pediatric labeling of
10 the drug or biological product in-
11 volved; and

12 “(II) in the case of such an inves-
13 tigation conducted with respect to
14 a combination of active ingredients
15 described to in clause (ii) of such sub-
16 paragraph, to assist in determining
17 the relevance of its molecular target
18 to the growth or progression of a pe-
19 diatric cancer.

20 “(iii) PRECLINICAL DATA.—The re-
21 ports on an investigation required in para-
22 graph (1)(B) shall include the results of all
23 preclinical studies on which the decision to
24 conduct such investigation was based.

1 “(iv) RULE OF CONSTRUCTION RE-
2 GARDING INACTIVE INGREDIENTS.—With
3 respect to a combination of active ingredi-
4 ents referred to in subparagraph (A)(ii),
5 such subparagraph may not be construed
6 as addressing the use of inactive ingredi-
7 ents with such combination. For purposes
8 of such subparagraph, the Secretary may
9 establish such requirements with respect to
10 inactive ingredients as the Secretary deter-
11 mines to be appropriate.”.

12 (2) CLARIFYING APPLICABILITY OF CERTAIN
13 PROVISIONS.—Section 505B(a)(3) of the Federal
14 Food, Drug, and Cosmetic Act (21 U.S.C.
15 355c(a)(3)), as amended by paragraph (1), is
16 amended by adding at the end the following:

17 “(E) INTERNAL COMMITTEE REVIEW; LA-
18 BELING CHANGES; DISSEMINATION OF INFOR-
19 MATION; ADVERSE EVENTS; SCOPE OF AUTHOR-
20 ITY.—Subsections (f) through (j) shall apply
21 with respect to investigations described in this
22 paragraph to the same extent and in the same
23 manner as such subsections apply with respect
24 to the assessments required under paragraph
25 (1)(A), except that subsection (g) does not

1 apply with respect to an investigation referred
2 to in subparagraph (A)(ii).”.

3 (3) CONFORMING AMENDMENTS.—Section
4 505B(a) of the Federal Food, Drug, and Cosmetic
5 Act (21 U.S.C. 355c(a)) is amended—

6 (A) in paragraph (3)(C), as redesignated
7 by paragraph (1)(A) of this subsection, by
8 striking “investigations described in this para-
9 graph” and inserting “investigations referred to
10 in subparagraph (A)(i)”;

11 (B) in paragraph (3)(D), as redesignated
12 by paragraph (1)(A) of this subsection, by
13 striking “the assessments under paragraph
14 (2)(B)” and inserting “the assessments re-
15 quired under paragraph (1)(A)”; and

16 (C) in paragraph (5)(D), by inserting be-
17 fore the period at the end the following: “, ex-
18 cept this subparagraph is not applicable to a
19 drug or biological product that is the subject of
20 an investigation referred to in paragraph
21 (3)(A)(ii)”.

22 (b) AUTHORITY REGARDING PRECLINICAL STUD-
23 IES.—Section 505B(a)(1) of the Federal Food, Drug, and
24 Cosmetic Act (21 U.S.C. 355c(a)(1)), as amended by sub-

1 section (b)(1), is further amended by adding at the end
2 the following:

3 “(D) PRECLINICAL STUDIES GEN-
4 ERALLY.—

“(i) IN GENERAL.—With respect to an application for an exemption for investigational use under section 505(i) for a drug or biological product that is intended for the treatment of an adult cancer, the Secretary may require, as a condition of permitting the exemption to go into effect, that the sponsor involved enter into an agreement with the Secretary to conduct not more than two preclinical studies of the drug or biological product in order to assist in determining the relevance of its molecular target to the growth or progression of a pediatric cancer.

19 “(ii) TIMEFRAME FOR PRECLINICAL
20 STUDIES.—With respect to the drug or bi-
21 ological product involved, an agreement
22 under clause (i) for a preclinical study
23 shall specify the date by which an initial
24 plan for the study will be submitted to the
25 Secretary. The results of the preclinical

1 study shall be submitted to the Secretary
2 in accordance with a timeframe to which
3 the Secretary and the sponsor involved
4 have agreed.”.

5 (c) APPLICABILITY.—The amendments made by this
6 section apply with respect to any application under section
7 505(i) of the Federal Food, Drug, and Cosmetic Act (21
8 U.S.C. 355(i)), any application under section 505 of such
9 Act (21 U.S.C. 355), and any application under section
10 351(a) of the Public Health Service Act (42 U.S.C. 262),
11 that is submitted on or after the expiration of the 3-year
12 period beginning on the date of the enactment of this Act.

